(19) World Intellectual Property **Organization**

International Bureau





(43) International Publication Date 9 December 2004 (09.12.2004)

PCT

(10) International Publication Number WO 2004/106488 A3

- (51) International Patent Classification⁷: C12Q 1/68, C12P 19/34, C12N 15/00, C07H 21/00, 21/02, 21/04
- (21) International Application Number:

PCT/US2003/014631

- (22) International Filing Date: 12 May 2003 (12.05.2003)
- (25) Filing Language:

English

(26) Publication Language:

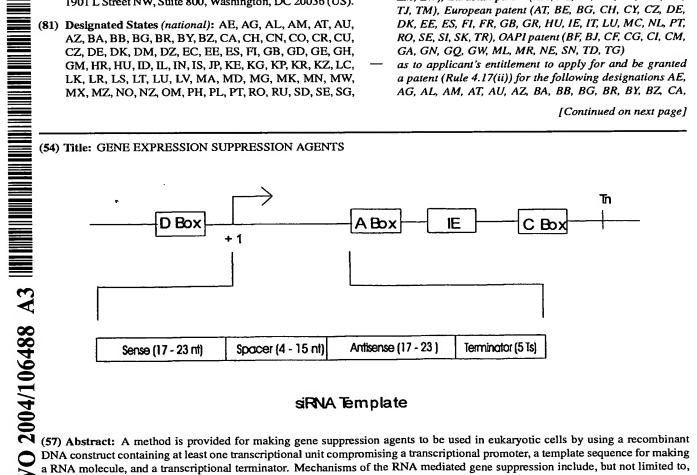
English

- (71) Applicant (for all designated States except US): PO-TOMAC PHARMACEUTICALS, INC. 11713 Tifton Drive, Potomac, MD 20854 (US).
- (75) Inventors/Applicants (for US only): YINGXIAN, Xiao [US/US]; 11713 Tifton Drive, Potomac, MD 20854 (US). XIN-HUA, Feng [CN/US]; 3119 Plumb Street, Houston, TX 77005 (US).
- (74) Agent: MOGA, Thomas, T.; Dickinson Wright PLLC, 1901 L Street NW, Suite 800, Washington, DC 20036 (US).
- (81) Designated States (national): AE, AG, AL, AM, AT, AU,

- SK, SL, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VN, YU, ZA, ZM, ZW.
- (84) Designated States (regional): ARIPO patent (GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian patent (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European patent (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PT, RO, SE, SI, SK, TR), OAPI patent (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

Declarations under Rule 4.17:

- as to the identity of the inventor (Rule 4.17(i)) for the following designations AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG. BR. BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NO, NZ, OM, PH, PL, PT, RO, RU, SD, SE, SG, SK, SL, TJ, TM, TN, TR, TT, TZ, UA, UG, UZ, VN, YU, ZA, ZM, ZW, ARIPO patent (GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian patent (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European patent (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PT,



DNA construct containing at least one transcriptional unit compromising a transcriptional promoter, a template sequence for making a RNA molecule, and a transcriptional terminator. Mechanisms of the RNA mediated gene suppression include, but not limited to, RNA interferences (RNAi). The use of the agents as tools for biomedical research as well as medicinal products is also disclosed.





CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NO, NZ, OM, PH, PL, PT, RO, RU, SD, SE, SG, SK, SL, TJ, TM, TN, TR, TT, TZ, UA, UG, UZ, VN, YU, ZA, ZM, ZW, ARIPO patent (GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian patent (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European patent (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PT, RO, SE, SI, SK, TR), OAPI patent (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG)

of inventorship (Rule 4.17(iv)) for US only

Published:

- with international search report
- before the expiration of the time limit for amending the claims and to be republished in the event of receipt of amendments

(88) Date of publication of the international search report: 17 March 2005

For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.

INTERNATIONAL SEARCH REPORT

International application No.

PCT/US03/14631

		101,000		
A. CLASSIFICATION OF SUBJECT MATTER				
IPC(7) : C12Q 1/68; C12P 19/34; C12N 15/00; C07H 21/00, 21/02, 21/04				
US CL: 435/6, 91.1, 91.5, 320.1; 536/23.1, 24.1, 25.3 According to International Patent Classification (IPC) or to both national classification and IPC				
B. FIELDS SEARCHED				
Minimum documentation searched (classification system followed by classification symbols) U.S.: 435/6, 91.1, 91.5, 320.1; 536/23.1, 24.1, 25.3				
Documentation searched other than minimum documentation to the extent that such documents are included in the fields searched				
Electronic data base consulted during the international search (name of data base and, where practicable, search terms used) West, Dialog				
C. DOCUMENTS CONSIDERED TO BE RELEVANT				
Category *	Citation of document, with indication, where ap	propriate, of the relevant passages	Relevant to claim No.	
Х	WONG-STAAL, F. Ribozyme gene therapy for HIV	infection intracellular immunization of	1, 2, 4, 6, 7, 9, 11-14	
	lymphocytes and CD34+ cells with an anti-HIV-1 ribozyme gene, advanced Drug Delivery Rev., Vol. 17, pates 363-368 (1995), p. 363, right col. on p. 364, text on p. 365. 3, 5, 8, 10			
Y	Rev., Vol. 17, pates 363-368 (1995), p. 363, right co	on p. 364, text on p. 363.	3, 3, 6, 10	
X		r Cell, Vol. 2, pages 617-627 (1998),	1, 2, 3, 5, 7, 8, 9,11- 14 4, 6, 10,	
Further	r documents are listed in the continuation of Box C.	See patent family annex.		
* Special categories of cited documents: "T" later document published after the international filing date or pridate and not in conflict with the application but cited to underst				
"A" document	document defining the general state of the art which is not considered to be principle or theory underlying the invention of particular relevance		ention	
"E" earlier ap	oplication or patent published on or after the international filing date	"X" document of particular relevance; the considered novel or cannot be conside when the document is taken alone	red to involve an inventive step	
"L" document which may throw doubts on priority claim(s) or which is cited to establish the publication date of another citation or other special reason (as specified)		"Y" document of particular relevance; the claimed invention cannot be considered to involve an inventive step when the document is combined with one or more other such documents, such combination		
"O" documen	ument referring to an oral disclosure, use, exhibition or other means being obvious to a person skilled in the art			
	ocument published prior to the international filing date but later than the "&" document member of the same patent family iority date claimed			
Date of the actual completion of the international search		Date of mailing of the martingtional search report		
08 December 2004 (08.12.2004)			16/	
Transcribe and the second of t		Authorized officer Music fation		
Mail Stop PCT, Atm: ISA/US Commissioner for Patents Jane Zara			/	
D.O. Den 1450				
1	Alexandria, Virginia 22313-1450			
Facsimile N	o. (703) 305-3230	l		